
Amyotrophic Lateral Sclerosis (ALS) Fact Sheet

CIRM funds many projects seeking to better understand ALS and to translate those discoveries into new therapies.

Description

About 6,000 people are diagnosed with ALS (also known as Lou Gehrig's disease) each year in the U.S., and the average survival time is two to five years. The disease results when the cells in the brain or spinal cord that instruct muscles to move—called motor neurons—die off. People with the disease lose the ability to move their muscles and, over time, the muscles atrophy and people become paralyzed and eventually die. There is no effective therapy for the disease.

California's stem cell agency has funded several research projects that could help people with ALS (the full list of CIRM awards in this disease is below). Some of those projects are very basic—researchers are trying to understand the origin of the disease and what causes the motor neurons to die. These are the kinds of questions researchers need to understand if they are going to develop the most effective therapies.

With CIRM funding, researchers have made progress understanding which cells are responsible for damaging the motor neurons. It turns out that the cells surrounding those neurons—called astrocytes—are secreting a chemical that damages the neurons. They've also learned how to take certain kinds of stem cells and turn them into motor neurons and astrocytes and this might help us better understand the relationship of these cells and even one day prove useful in developing new ways to treat people with ALS.

We also fund projects that are in the later stages of research leading up to and in some cases including clinical trials. These projects involve teams of researchers who carry out the experiments that are required before the U.S. Food and Drug Administration will allow the potential therapy to be tested in people. Recently, CIRM has funded research into ALS that has advanced into clinical trials. You can read more about these trials below.

Clinical Stage Programs

Cedars-Sinai Medical Center

This team of researchers plans to protect surviving neurons in people diagnosed with ALS from further degeneration. They will implant middle-man "progenitor" cells made by maturing stem cells from fetal tissue down a path destined to become astrocytes, the brain cells that protect nerves and that become defective in ALS. Those cells will be boosted with genes for a growth factor that when the cells release it after transplantation, will have an added protective effect on nerves. This approach recently received approval to treat ALS patients in a CIRM-funded clinical trial (read here). A feature story on this work appeared in The Stem Cellar blog in 2017.

- [Learn more about this clinical project](#)
- [For more information about this clinical trial, click here.](#)

Brainstorm Cell Therapeutics

BrainStorm is using mesenchymal stem cells that are taken from the patient's own bone marrow to treat patients with ALS. These stem cells are then modified to boost their production of neurotrophic factors, which are known to help support and protect neurons, the cells destroyed by the disease. The CIRM funding will enable the company to test this therapy, called NurOwn, in a Phase 3 trial involving around 200 patients.

- [Learn more about this clinical project](#)
- [For more information about this clinical trial, click here.](#)















CIRM Grants Targeting ALS

Researcher name	Institution	Grant Title	Grant Type	Approved funds	
Lawrence Goldstein	University of California, San Diego	Human Embryonic Stem Cell-Derived Neural Stem Cell Transplants in Amyotrophic Lateral Sclerosis	Therapeutic Translational Research Projects	\$5,590,578	
Samuel Pfaff	Salk Institute for Biological Studies	Gene regulatory mechanisms that control spinal neuron differentiation from hES cells.	SEED Grant	\$704,543	
John Ravits	University of California, San Diego	California ALS Research Summit 2016	Conference II	\$11,400	
Justin Ichida	University of Southern California	The 7th Annual California ALS research network and PAC10 meeting	Conference II	\$10,830	
Clive Svendsen	Cedars-Sinai Medical Center	Human Neural Progenitors Secreting Glial Cell Line-Derived Neurotrophic Factor (CNS10-NPC-GDNF) for the Treatment of Amyotrophic Lateral Sclerosis	Clinical Trial Stage Projects	\$6,154,067	
Ralph Kern	BrainStorm Cell Therapeutics	A Phase 3, Randomized, Placebo-controlled Multicenter Study to Evaluate Efficacy & Safety of Repeated Administrations of NurOwn® in Patients with ALS	Clinical Trial Stage Projects	\$15,912,390	
Clive Svendsen	Cedars-Sinai Medical Center	Progenitor Cells Secreting GDNF for the Treatment of ALS	Disease Team Therapy Development - Research	\$16,168,464	
Bennett Novitch	University of California, Los Angeles	Molecular Characterization of hESC and hiPSC-Derived Spinal Motor Neurons	Basic Biology I	\$1,229,922	
Eugene Yeo	University of California, San Diego	Stem cell models to analyze the role of mutated C9orf72 in neurodegeneration	Basic Biology IV	\$1,260,360	
Lawrence Goldstein	University of California, San Diego	Stem Cell-Derived Astrocyte Precursor Transplants in Amyotrophic Lateral Sclerosis	Disease Team Research I	\$5,694,308	
Steven Finkbeiner	Gladstone Institutes	Development of Novel Autophagy Inducers to Block the Progression of and Treat Amyotrophic Lateral Sclerosis (ALS) and Other Neurodegenerative Diseases	Early Translational IV	\$2,049,053	
Zack Jerome	University of California, Los Angeles	Generation of clinical grade human iPS cells	New Cell Lines	\$1,341,000	
Lawrence Goldstein	University of California, San Diego	Stem Cell-Derived Astrocyte Precursor Transplants in Amyotrophic Lateral Sclerosis	Early Translational from Disease Team Conversion	\$4,139,754	

Fred Gage	Salk Institute for Biological Studies	Development of Induced Pluripotent Stem Cells for Modeling Human Disease	New Cell Lines	\$1,737,720	
Eric Ahrens	University of California, San Diego	Molecular Imaging for Stem Cell Science and Clinical Application	Research Leadership	\$5,920,899	
Binhai Zheng	University of California, San Diego	Genetic manipulation of human embryonic stem cells and its application in studying CNS development and repair	SEED Grant	\$600,441	
Leif Havton	University of California, Los Angeles	Development of a Relevant Pre-Clinical Animal Model as a Tool to Evaluate Human Stem Cell-Derived Replacement Therapies for Motor Neuron Injuries and Degenerative Diseases	Tools and Technologies III	\$1,308,711	
Bin Chen	University of California, Santa Cruz	In vitro differentiation of hESCs into corticospinal motor neurons	SEED Grant	\$465,624	
Steven Finkbeiner	Gladstone Institutes	THE 5TH ANNUAL CALIFORNIA ALS PAC10 AND RESEARCH NETWORK MEETING	Conference	\$9,529	
Jeremy Reiter	University of California, San Francisco	High throughput modeling of human neurodegenerative diseases in embryonic stem cells	New Faculty II	\$2,259,092	
Martina Wiedau-Pazos	University of California, Los Angeles	California ALS Summit 2011	Conference	\$13,300	
Hans Keirstead	University of California, Irvine	hESC-Derived Motor Neurons For the Treatment of Cervical Spinal Cord Injury	Comprehensive Grant	\$2,158,445	
Clive Svendsen	Cedars-Sinai Medical Center	California ALS Summit 2012	Conference	\$6,825	
Fred Gage	Salk Institute for Biological Studies	Molecular and Cellular Transitions from ES Cells to Mature Functioning Human Neurons	Comprehensive Grant	\$2,749,293	
John Ravits	University of California, San Diego	Cell Therapy for amyotrophic Lateral Sclerosis (ALS) - Testing the Limits: What should we use as preclinical standards of clinical trials?	Conference	\$7,193	
Ying Liu	University of California, San Diego	Generation of disease models for neurodegenerative disorders in hESCs by gene targeting	Tools and Technologies I	\$709,829	
Bin Chen	University of California, Santa Cruz	Molecular mechanisms of neural stem cell differentiation in the developing brain	New Faculty I	\$2,147,592	
Eugene Yeo	University of California, San Diego	Neural and general splicing factors control self-renewal, neural survival and differentiation	Basic Biology III	\$1,287,619	

Clive Svendsen	Cedars-Sinai Medical Center	Stem Cells Secreting GDNF for the Treatment of ALS	Disease Team Therapy Planning I	\$63,487	
Eugene Yeo	University of California, San Diego	Molecules to Correct Aberrant RNA Signature in Human Diseased Neurons	Early Translational III	\$1,532,323	
					Total: \$83,244,591.00

CIRM ALS Videos

 <p>Stem Cells in Your Face: Treating ALS with a Disease in a Dish</p>	 <p>Google Hangout: Progress Toward Stem Cell Therapies for ALS</p>	 <p>Webinar: Injecting Neural Stem Cells into ALS Patients: Results of a Phase 1 Trial Jonathan Glass</p>	 <p>\$18 Million ALS Research Grant for Stem Cell Therapy Applauded by ALS Advocates</p>
 <p>Lou Gehrig's Disease (ALS): Progress and Promise in Stem Cell Research</p>	 <p>Spotlight on Disease Team Awards - ALS: Introduction</p>	 <p>Spotlight on Disease Team Awards - ALS: Larry Goldstein</p>	 <p>Spotlight on Disease Team Awards - ALS: Don Cleveland</p>
 <p>Spotlight on Disease Team Awards - ALS: Mark Bonyhadi</p>	 <p>Spotlight on Disease Team Awards - ALS: Martin Marsala</p>	 <p>Spotlight on Disease Team Awards - ALS: Lucie Bruijn</p>	 <p>Spotlight on Disease Team Awards - ALS: Dan Desmond</p>
 <p>Spotlight on Disease Team Awards - ALS: Wrap Up</p>	 <p>Stem Cell Research Today: Larry Goldstein - CIRM Science Writer's Seminar</p>		

News and Information

- Can Stem Cell Therapies Help ALS Patients?
- *CIRM Stem Cellar Blog* on ALS research
- Stories of Hope: ALS

Resources

- CDC: ALS Information
- NIH: ALS Fact Sheet
- National Organization for Rare Disorders: ALS
- Find a clinical trial near you: NIH Clinical Trials database
- ALS Association
- ALS Foundation for Life
- ALS Hope Foundation
- Family Caregiver Alliance
- National Family Caregivers Association

Find Out More:

[Stem Cell FAQ](#) | [Stem Cell Videos](#) | [What We Fund](#)

Source URL: <https://www.cirm.ca.gov/our-progress/disease-information/amyotrophic-lateral-sclerosis-als-fact-sheet>